(19) World Intellectual Property Organization

International Bureau





(10) International Publication Number

PC

(43) International Publication Date 1 September 2005 (01.09.2005)

(51) International Patent Classification⁷: C12N 15/869, 15/86, A61K 48/00, C12N 5/10

(21) International Application Number:

PCT/US2005/005461

(22) International Filing Date:

17 February 2005 (17.02.2005)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

60/545,375

17 February 2004 (17.02.2004) US

(71) Applicant (for all designated States except US): UNI-VERSITY OF FLORIDA RESEARCH FOUNDA-TION, INC. [US/US]; 223 Grinter Hall, P.O. Box 115500, Gainsville, FL 32611-5500 (US).

(72) Inventors; and

- (75) Inventors/Applicants (for US only): BLOOM, David,
 C. [US/US]; 2302 NW 15th Place, Gainesville, FL 32605
 (US). AMELIO, Antonio, L. [US/US]; 4139 NW 34th
 Terrace, Gainesville, FL 32605 (US).
- (74) Agent: MOORE, Mark, D.; Haynes and Boone, L.L.P., 901 Main Street, Suite 3100, Dallas, TX 75202 (US).

WO 2005/080581 A3

- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SM, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

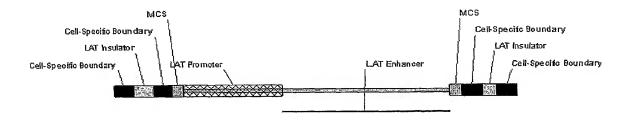
Published:

with international search report

(88) Date of publication of the international search report: 27 April 2006

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: INSULATED HERPESVIRUS-DERIVED GENE EXPRESSION CASSETTES FOR SUSTAINED AND REGULATABLE GENE EXPRESSION



Gene Expression Cassette

3646 bp

(57) Abstract: Disclosed are genetic expression cassettes, and vector comprising them useful for the delivery of nucleic acid segments encoding selected therapeutic constructs (including for example, peptides, polypeptides, ribozymes, and catalytic RN molecules), to selected cells and tissues of vertebrate animals. The disclosed genetic constructs are useful in the development of gene therapy vectors, including for example, viral vectors such as HSV, retroviral, lentiviral, AV, and rAAV vectors. The expression cassettes disclosed herein provide new tools in the field of gene therapy, and for the treatment of mammalian, and in particular, human diseases, disorders, and dysfunctions. The disclosed compositions may be utilized in a variety of investigative, diagnostic and therapeutic regimens, including the prevention and treatment of a variety of human diseases.

